

Adeno-Associated Virus Vectors
Encoding Factor VIII and Methods of Using the Same

Abstract

5 The present invention provides recombinant adeno-associated virus (rAAV)
vectors comprising a heterologous nucleotide sequence encoding factor VIII (factor
VIII). In preferred embodiments, the factor VIII is a B-domain deleted factor VIII. Also
provided are methods of producing a high titer stock of the inventive rAAV/factor VIII
vectors. Another aspect of the invention is a method of delivering a nucleotide sequence
10 encoding factor VIII to a cell, preferably for subsequent administration to a subject. The
present invention further provides methods of administering rAAV/factor VIII to a
subject, *e.g.*, for the treatment of hemophilia. The rAAV vector may be administered by
any route, but is preferably administered to the liver.